

CIRM Funded Clinical Trials

Clinical Trial of Stem Cell Gene Therapy for Sickle Cell Disease

Disease Area:	Sickle Cell Disease
Investigator:	Donald Kohn
Institution:	University of California, Los Angeles
CIRM Grant:	DR3-06945
Award Value:	\$13,145,465
Trial Sponsor:	University of California, Los Angeles
Trial Stage:	Phase 1
Trial Status:	Recruiting
Targeted Enrollment:	6
ClinicalTrials.gov ID:	NCT02247843



Donald Kohn

Details:

Sickle cell disease (SCD) is caused by a genetic mutation in the hemoglobin gene which causes red blood cells to "sickle" under conditions of low oxygen. SCD affects 1:500 African-Americans and is also common in Hispanic-Americans. The median survival for patients with SCD is 42 years for males and 48 years for females. A team at UCLA is genetically modifying a patient's own blood stem cells to produce a correct version of hemoglobin, the protein that is mutated in these patients, which causes abnormal sickle-like shaped red blood cells. These misshapen cells lead to dangerous blood clots, debilitating pain and even death. The genetically modified stem cells will be given back to the patient to create a new sickle cell-free blood supply.

Design:

Open label, single arm study.

Goal:

Primary: Safety, feasibility. Secondary: Hematopoietic Recovery; RBC function; Quality of life assessment.

Updates:

Enrolling.

News Releases:

Defeating Sickle Cell Disease with Stem Cells + Gene Therapy

[Contact Trial Sponsor](#)

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